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OHSU and Aptose Present New CG-806 Preclinical Data at ASH 60th Annual Meeting

CG-806 Pan-FLT3/Pan-BTK Inhibitor Exhibits Favorable Safety Profile and Unexpected Potency on Primary Cells from B-cell Cancer Patients and AML Patients

PORTLAND, Ore. and SAN DIEGO, Dec. 03, 2018 (GLOBE NEWSWIRE) -- Oregon Health & Science University (OHSU) and Aptose Biosciences Inc. (NASDAQ: APTO, TSX: APS) today announced the presentation of preclinical data demonstrating that CG-806, a first-in-class pan-FLT3/pan-BTK inhibitor, exhibits broad ex vivo potency on bone marrow cells from patients with diverse hematologic malignancies and superior potency relative to ibrutinib on those bone marrow cells from patients with CLL or other B-cell cancers. In addition, bioinformatic analyses revealed an unexpected ex vivo potency of CG-806 on bone marrow cells from AML patients with IDH1 mutations or with FLT3-ITD mutations. Plus, CG-806 demonstrated a favorable safety profile in GLP toxicology and safety studies. The data were highlighted in a poster presentation on Sunday, December 2, 2018 at the 60th American Society of Hematology (ASH) Annual Meeting and Exposition being held December 1-4, 2018 in San Diego, CA. Separately, Aptose and The University of Texas MD Anderson Cancer Center researchers also presented new data on CG-806 at ASH (see press release [here](#)).

Studies have shown that more than 50% of patients with CLL and mantle cell lymphoma (MCL) discontinue ibrutinib treatment due to intolerance or emergence of resistant disease. "Our data indicate that CG-806 clearly addresses ibrutinib's shortcomings, inhibiting driver and rescue pathways to directly and potently kill a broad range of malignant B-cells. CG-806 has potential to be an important part of our future armamentarium against hematologic malignancies that are resistant, refractory or intolerant to other therapies," said Jeffrey W. Tyner, Ph.D., Associate Professor in the OHSU School of Medicine department of Cell, Developmental & Cancer Biology.

The poster, [**CG-806, a First-in-Class Pan-FLT3/Pan-BTK Inhibitor, Exhibits Broader and Greater Potency than Ibrutinib Against Primary and Cultured Malignant B Cells**](#), evaluated the activity of CG-806 on various hematologic malignancy cell lines and patient primary bone marrow specimens. CG-806 inhibited cell proliferation and induced apoptosis with a potency that was 50-6,000 times greater than that of ibrutinib when tested against 14 established malignant B-cell lines *in vitro*. When tested against 124 samples freshly isolated from the bone marrow of chronic lymphocytic leukemia (CLL) patients, the median IC50 for CG-806 was 0.11 μ M and the median for ibrutinib was 4.09 μ M, respectively, $p < 0.001$. Since stromal mediated signaling plays an important role in malignant B-cell survival and chemoresistance, the apoptotic effect of CG-806 was further analyzed on cultured and

primary malignant B-cells in the presence of stromal cells, and its potency was not impaired. CG-806 was shown to be significantly more potent than Ibrutinib at inhibiting malignant B-cell colony formation, migration, and inducing apoptosis in the presence of stromal cells.

Primary cells from patients with diverse hematologic malignancies are highly sensitive to CG-806, including cells with the FLT3-ITD mutation, found in approximately 30% of acute myeloid leukemia (AML) patients. The data presented at ASH showed that primary cells from AML patients with the IDH-1 mutation similarly demonstrated high sensitivity to CG-806. Aptose also reported new results from the 28-day GLP toxicity and toxicokinetic studies of CG-806, which continue to demonstrate a highly favorable safety profile with no adverse findings to date.

“CG-806’s activity against IDH-1 mutant AML patient bone marrow cells is an unexpected new finding that further broadens its potential use and potential paths for rapid development,” said William G. Rice, Ph.D., Chairman and Chief Executive Officer of Aptose. “In addition, CG-806 has continued to perform well in all toxicology and safety studies, and we look forward to filing an IND in early 2019.”

About CG-806

CG-806 is a preclinical stage oral, first-in-class pan-FLT3/pan-BTK multi-cluster kinase inhibitor. This small molecule demonstrates potent inhibition of wild type and all mutant forms of FLT3 (including internal tandem duplication, or ITD, and mutations of the receptor tyrosine kinase domain and gatekeeper region), eliminates acute myeloid leukemia (AML) tumors in the absence of toxicity in murine xenograft models, and represents a potential best-in-class therapeutic for patients with AML. Likewise, CG-806 demonstrates potent, non-covalent inhibition of the wild type and Cys481Ser mutant forms of the BTK enzyme, as well as other oncogenic kinase pathways operative in B cell malignancies, suggesting CG-806 may be developed for various B-cell malignancy patients (including CLL, MCL, DLBCL and others) that are resistant/refractory/intolerant to covalent BTK inhibitors. It is in development for acute myeloid leukemia (AML) and B cell lymphoma.

About Aptose

Aptose Biosciences is a clinical-stage biotechnology company committed to developing personalized therapies addressing unmet medical needs in oncology, with an initial focus on hematology. The company's small molecule cancer therapeutics pipeline includes products designed to provide single agent efficacy and to enhance the efficacy of other anti-cancer therapies and regimens without overlapping toxicities. APTO-253, the only clinical stage agent that directly targets the MYC oncogene and inhibits its expression, is in a Phase 1b clinical trial for the treatment of patients with relapsed or refractory acute myeloid leukemia (AML) or high risk MDS. CG-806 is an oral, first-in-class pan-FLT3/pan-BTK multi-cluster kinase inhibitor being developed to treat AML and certain B cell malignancies. For further information, please visit www.aptose.com.

Forward Looking Statements

This press release contains forward-looking statements within the meaning of Canadian and U.S. securities laws, including, but not limited to, statements regarding our intentions or current expectations concerning, among other things, the anti-tumor activity of CG-806, the clinical potential and favorable properties of CG-806, the IND filing clinical trials for CG-806 and their expected timing, and statements relating to the Company’s plans, objectives, expectations and intentions and other statements including words such as “continue”,

“expect”, “intend”, “will”, “should”, “would”, “may”, and other similar expressions. Such statements reflect our current views with respect to future events and are subject to risks and uncertainties and are necessarily based upon a number of estimates and assumptions that, while considered reasonable by us are inherently subject to significant business, economic, competitive, political and social uncertainties and contingencies. Many factors could cause our actual results, performance or achievements to be materially different from any future results, performance or achievements described in this press release. Such factors could include, among others: our ability to obtain the capital required for research and operations; the inherent risks in early stage drug development including demonstrating efficacy; development time/cost and the regulatory approval process; the progress of our clinical trials; our ability to find and enter into agreements with potential partners; our ability to attract and retain key personnel; changing market and economic conditions; inability of new manufacturers to produce acceptable batches of GMP in sufficient quantities; unexpected manufacturing defects; and other risks detailed from time-to-time in our ongoing quarterly filings, annual information forms, annual reports and annual filings with Canadian securities regulators and the United States Securities and Exchange Commission.

Should one or more of these risks or uncertainties materialize, or should the assumptions set out in the section entitled "Risk Factors" in our filings with Canadian securities regulators and the United States Securities and Exchange Commission underlying those forward-looking statements prove incorrect, actual results may vary materially from those described herein. These forward-looking statements are made as of the date of this press release and we do not intend, and do not assume any obligation, to update these forward-looking statements, except as required by law. We cannot assure you that such statements will prove to be accurate as actual results and future events could differ materially from those anticipated in such statements. Investors are cautioned that forward-looking statements are not guarantees of future performance and accordingly investors are cautioned not to put undue reliance on forward-looking statements due to the inherent uncertainty therein.

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